Clinical Study to Assess Safety and Efficacy of Subretinal Injection of Human Neural Progenitor Cells for Treatment of Retinitis Pigmentosa

Grant Award Details

Clinical Study to Assess Safety and Efficacy of Subretinal Injection of Human Neural Progenitor Cells for Treatment of Retinitis Pigmentosa

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-11620

Investigator:

Name:	Clive Svendsen
Institution:	Cedars-Sinai Medical Center
Type:	PI

Disease Focus: Retinitis Pigmentosa, Vision Loss

Human Stem Cell Use: Adult Stem Cell

Award Value: $10,494,682

Status: Pre-Active

Grant Application Details

Application Title: Clinical Study to Assess Safety and Efficacy of Subretinal Injection of Human Neural Progenitor Cells for Treatment of Retinitis Pigmentosa

Public Abstract:

Therapeutic Candidate or Device

CNS10-NPC - a human neural progenitor cell line

Indication

Retinitis Pigmentosa

Therapeutic Mechanism

1. Phagocytosis of photoreceptor outer segment debris.
2. The release of pro-survival factors that have localized diffusion to inhibit retinal photoreceptor cell death.
3. Immunomodulation resulting in markedly fewer host inflammatory cells at the site of CNS10-NPC engraftment

Unmet Medical Need

Retinitis pigmentosa represents an unmet clinical need in ophthalmology. Despite growing understanding of the underlying molecular mechanisms, there remains little in the way of available treatment.

Project Objective

Phase 1/2a Completed

Major Proposed Activities

- Assess clinical safety of the clinical product (CNS10-NPC)
- Obtain clinical data based on secondary outcome measures of vision loss
- Manufacture additional clinical product for a subsequent Phase 2 trial

Statement of Benefit to California:

There are over 10,000 retinitis pigmentosa patients in CA who could benefit from this type of stem cell treatment. The information gained through this trial will also advance the field of cell therapy for this disease. While gene therapy is a promising approach for patients with specific mutations, cell-based therapies have the potential to be applicable to all retinitis pigmentosa patients regardless of genotype. If successful, this therapy could also benefit patients with macular degeneration.

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